NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

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EC Grants Orphan Drug Designation to viltolarsen (NS-065/NCNP-01)

Kyoto, Japan, June 8, 2020 – Nippon Shinyaku Co., LTD. (Nippon Shinyaku; Kyoto; President, Shigenobu Maekawa) announced that European Commission (EC) has granted Orphan Drug Designation to viltolarsen (NS-065/ NCNP-01) being studied for the treatment of Duchenne muscular dystrophy (DMD).

DMD is a progressive muscle disorder in which deletion or mutation of part of the dystrophin gene results in loss of normal dystrophin protein production and muscle weakness, mostly in boys. Viltolarsen is an antisense oligonucleotide designed to help produce dystrophin protein that contains essential functional portions in patients with DMD mutations amenable to exon 53 skipping by skipping exon 53. VILTEPSO® (viltolarsen) Intravenous Infusion 250 mg was launched in Japan in May, this year. The NDA for viltolarsen is also being reviewed under an accelerated approval pathway in the U.S. The clinical study in the US is being conducted by NS Pharma, Inc. (Paramus NJ, USA; President, Tsugio Tanaka), a wholly owned subsidiary of Nippon Shinyaku.

The Orphan Drug Designation by EC is issued to drugs which are intended for diseases which affect fewer than 5 in 10,000 people in European Union (EU), and are life-threatening or chronically debilitating. If approved, the designation provides for a ten-year marketing exclusivity period. Viltolarsen also received various designations by the Ministry of Health, Labour and Welfare (MHLW) in Japan and by the FDA in the U.S. This is the first designation for viltolarsen in the EU.

Nippon Shinyaku's mission is to develop treatments for intractable and rare diseases, and offer new options for patients suffering from DMD worldwide.

Viltolarsen (NS-065/NCNP-01)

Viltolarsen is a morpholino antisense oligonucleotide which was co-discovered by Nippon Shinyaku and National Center of Neurology and Psychiatry (NCNP: Kodaira City, Tokyo; President, Hidehiro Mizusawa). It is designed to produce a shortened dystrophin protein that contains essential functional portions in patients with DMD mutations amenable to exon 53 skipping by skipping exon 53.

Designations relating to viltolarsen

<From MHLW>

- "SAKIGAKE designation (Japanese version of Breakthrough Therapy Designation) " in October, 2015
- Orphan Disease designation in August, 2019
- Designation subjected to Conditional Early Approval System in October, 2019

<From FDA>

- Fast Track designation in October, 2016
- Orphan drug designation and Rare Pediatric Disease designation in January, 2017
- Priority Review in January 2020

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